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This report includes selected news items from the past week on issues of concern to the bleeding disorders community. It is designed to help keep NHF national and local leadership and staff informed of the latest information from the news media. It will be distributed by email on Thursday of each week, covering important news items from the previous seven days. Subjects covered will include hemophilia, other bleeding disorders, gene therapy, hepatitis, HIV/AIDS, and others.

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July 20, 2018
Endpts.com

Roche Has Taken the Trump Pledge, Joining Pfizer, Novartis and Merck in Promising to Hold the Line on Drug Prices

By John Carroll

You can add yet another Big Pharma to the growing list of giants swearing off US price hikes.

Roche tells me this morning that they told HHS on July 11 — the day after Pfizer CEO Ian Read agreed to roll back a price hike in a call with the president — that “we will not be taking any price increases for the remainder of the year.”

Roche’s move comes as Pfizer, Novartis and Merck have all pledged to hold the line on drug prices for the rest of the year — which comes amid considerable skepticism over just how meaningful this is. And Reuters is reporting that Bayer and Merck KGaA are also falling into line.

Roche, though, almost immediately was called out for freezing prices after they had already completed two rounds of price hikes this year. Bloomberg reports that the pharma giant raised prices on its big three cancer drugs — Avastin, Perception and Rituxan — and others just days ago, following increases in January.

Based on previous years, Roche may well have been finished raising prices anyway.

The move will surely lead to plenty of new hoots from critics, who have been calling out Merck for slashing the price of a hep C therapy that was all but dead anyway, along with other drugs that had lost patent protection.

Impactful or not, from the perspective of the Trump administration, this is another big win. The optics on these price freezes are just what Donald Trump was looking for when he called up Pfizer CEO Ian Read following the company’s move to raise prices on dozens of products.

Now it looks like we’ll soon have a majority of the top 10 promising to hold the line, at least for the rest of this year.

Here’s the rest of the statement from Roche:

We take decisions related to the prices of our medicines very seriously and our commitment to patient access and investment in future breakthroughs are reflected in our actions. This applies to the way we price new medicines and how we change the price of those medicines over time.

For example, our last seven new medicines were priced less than other approved medicines used to treat a similar disease. We recently priced Ocrevus, an innovative treatment for multiple sclerosis, 25% lower than the medicine it surpassed in clinical trials, and Hemlibra at less than half the cost of the standard treatment for hemophilia A with inhibitors.

Also, over the past several years, Genentech’s annual average net price increase, weighted by sales, was approximately 3% – in line with the medical consumer price index (CPI).

But for patients and the healthcare system to benefit fully from responsible pricing actions, we must also focus on implementing long-term, system-wide solutions that lower costs, while sustaining scientific innovation and access to life-changing medicines.

To that end, we are engaging with HHS and others to propose and discuss ideas and recommendations that can bring about positive, lasting change. These include enabling the Centers for Medicare & Medicaid Services to benefit more directly from private sector competition for Part B medicines; reforming the 340B program; and implementing novel pricing and reimbursement models.

We're committed to being part of the solution and look forward to continuing these discussions.

July 23, 2018
Businesswire.com

Individuals with Hemophilia Can Now Receive Financial Support from The Assistance Fund

New financial assistance program provides eligible patients support for medication copayments, health insurance premiums and incidental medical expenses

The Assistance Fund, an independent charitable patient assistance foundation that helps patients and families facing high medical out-of-pocket costs, today announced the launch of a new financial assistance program for individuals with hemophilia. The program is designed to help qualified patients pay for medication copayments, coinsurance and deductibles, health insurance premiums and incidental medical expenses.

Hemophilia is a rare inherited disorder caused by a mutation in the gene responsible for blood clotting. The blood's inability to clot leads to excessive bleeding following injuries or surgeries and, in severe cases, bleeding in the brain can cause long term problems such as seizures or paralysis.¹ It is more common for males to have the condition. Currently, about 20,000 males in the United States are living with hemophilia.¹

“For people with hemophilia, even routine activities, like going to the dentist, can be daunting,” said Mark P. McGreevy, President and CEO, The Assistance Fund. “The opening of TAF’s Hemophilia Financial Assistance Program will provide financial support to patients so that they can better afford the treatments they need to help minimize bleeding episodes and improve their quality of life.”

Aside from excessive bleeding, people with hemophilia can also experience chronic joint disease and pain, large bruises or irregular nosebleeds.² Common treatments include clotting factor injections, called replacement therapy, as a prophylactic measure or “on demand therapy” following an injury.²

To learn more or determine eligibility for financial support, individuals should visit tafcares.org or call 844-282-5802 to speak with a patient advocate.

A list of all the programs available at The Assistance Fund can be found on the website tafcares.org.

About The Assistance Fund

The Assistance Fund is an independent charitable patient assistance foundation that helps patients and families facing high medical out-of-pocket costs by providing financial assistance for their copayments, coinsurance, deductibles and other health-related expenses. The Assistance Fund currently manages more than 40 funds – each of which covers the FDA-approved medications that treat a specific disease. Since its founding in 2009, The Assistance Fund has helped more than 73,000 adults and children access the medicines they need to stay healthy or manage a chronic condition. To learn more about The Assistance Fund, or for information on how to donate, please visit tafcares.org.

References

1. Basics About Hemophilia. Centers for Disease Control and Prevention. <https://www.cdc.gov/ncbddd/hemophilia/facts.html>. Revised May 4, 2018. Accessed July 11, 2018.

2. Hemophilia. National Heart, Lung, and Blood Institute. <https://www.nhlbi.nih.gov/health-topics/hemophilia>. Accessed July 11, 2018.

July 23, 2018
Madison.com

University of Wisconsin Pianist Shares Musical Gift Despite Health Challenge

By Samara Kalk Derby

For the most part, audiences who watch Kangwoo Jin's fingers fly around the piano keys have no indication that he suffers from a rare bleeding disorder that can cause terrible pain in his joints.

Kangwoo, who got a not-so-rare standing ovation after a vigorous recent performance of Chopin's Étude Op. 10, No. 4, was diagnosed with severe hemophilia as an infant in South Korea.

He has to give himself injections of factor VIII — an essential blood-clotting protein — two or more times a week to keep his bleeding under control.

Kangwoo is a doctoral student in piano at UW-Madison and a teacher who's won at least 10 prestigious competitions. He's performed as a soloist with the UW Symphony Orchestra and on Wisconsin Public Radio.

"I'm always thankful that I can still play the piano and make the music I can," said Kangwoo, 35, who began playing piano at age 7 in South Korea. "I'm more hopeful and try to be positive."

Kangwoo's UW-Madison piano professor, Christopher Taylor, said that, amazingly, he had little awareness at first of Kangwoo's condition, since Kangwoo never complained.

"But gradually I came to realize how formidable the obstacles are that he has had to contend with, and my admiration for his persistence and fortitude grows continually."

Taylor said Kangwoo has proved to be an outstanding student in the four years since he arrived in his studio, "always playing with passion and commitment, and always eager to participate to the fullest in the School of Music community, both as a soloist and as a chamber musician."

In addition, he also credits Kangwoo as a "dynamic and sophisticated teacher."

But without regular medication, Kangwoo could have a life-threatening bleed, said Stephanie Lovell, his hematology nurse practitioner in the UW Health Comprehensive Program for Bleeding Disorders. She said bleeds happen mostly inside of muscles and joints, which isn't how many people think of hemophilia.

Hemophilia is a rare genetic bleeding disorder that slows the blood-clotting process. It occurs much more commonly in males than in females. According to the Centers for Disease Control and Prevention, about 400 babies are born with hemophilia A each year.

The exact number of people living with hemophilia in the U.S. is estimated to be about 20,000, based on expected births and deaths since 1994.

Lovell said the life expectancy for a person with hemophilia is now the same as the general population, which is a relatively recent development. While treatments in the U.S. are working well, there are still lots of barriers that make life with hemophilia difficult.

“Because people are living longer with hemophilia than they did in the past, we are encountering lots of new, uncharted territory,” she said.

The ‘real deal’

It’s widely believed that hemophiliacs bleed profusely if they get a cut or a scratch. The reality is that external wounds aren’t usually serious. Internal bleeding, meanwhile, comes from overuse, injury or can happen spontaneously, said Lovell, who has worked with Kangwoo since he came to UW from the University of Indiana, where he got his master’s degree in 2014. He’s been in the United States since 2009.

“It’s twisting an ankle or doing repetitive movements that causes bleeding inside of a joint, and once you have one bleed inside that joint, you’re much more likely to have more because it’s irritated and inflamed and then the vessels break more easily,” she said.

All of Kangwoo’s joint damage is permanent, with his right elbow, left knee and left ankle suffering the most. But being on a regular medication schedule keeps it from getting worse and allows him to lead a busy life, he said.

Kangwoo is limited when it comes to physical activity. He can’t hike or camp and it hurts him to walk for more than 15 minutes. Once bleeding happens in one of his joints, it can aggravate the joint sometimes even when he has recently injected his medication. With regular injections, he’s able to bike for half an hour, which he does regularly.

To show his appreciation for the care he’s received, Kangwoo played a short concert Wednesday for Lovell and others from the clinic who’ve taken care of him. He performed on a baby grand piano in the UW Hospital atrium.

Jessica Johnson, Kangwoo’s other primary piano professor at UW-Madison, said Kangwoo’s love of life and passion for sharing music with others makes the world a better place.

“He pursues his art with wholehearted joy, love, and an unbridled enthusiasm that is contagious,” Johnson said. “I think that the challenges he has faced have made him a more generous, compassionate and accepting person.”

Lovell said she was fortunate to attend one of Kangwoo’s recent performances in addition to Wednesday’s.

“He’s the real deal,” she said. “It was fantastic. It was very impressive, and I just felt very proud that I knew him and know of the challenges he’s overcome.”

Hope for others

Hemophilia can cause bleeding in Kangwoo’s right elbow, which he said is not in a great shape.

“It’s already deforming. I cannot bend it,” he said.

If he has a recital or a competition coming up, he might practice five hours a day and when he plays loudly, it can hurt, he said.

“So I have to be really careful. I have to not practice as much as I can. It can possibly cause a bleeding episode in my elbow. So, I practice those the day I infuse.”

Treatment for hemophilia used to be reactive, so that patients were given medicine when a bleed happened. Now it’s preventative.

Outwardly, no one would know of Kangwoo’s condition, but Lovell stresses how difficult it is for him to have to stick himself in a vein two or three times a week. For instance, insulin, used to manage diabetes, is easier, because diabetics don’t need to find a vein; they inject the insulin into the tissue below the skin.

Parents or caregivers need to inject hemophiliac children with factor VIII so they can lead regular lives, and that’s why Lovell is eager to share Kangwoo’s story.

Hemophilia drugs are among the most expensive on the market and can cost about \$3,000 per dose. With procedures and other care, the cost of managing the disorder can reach a million dollars a year, Lovell said.

In the U.S., patients are generally able to get the treatment they need through commercial or government-sponsored health insurance, along with other assistance programs that help cover some of their out-of-pocket expenses.

Until 2009, Kangwoo had Korean government insurance and his family didn’t have to pay much, he said. “That’s probably why there is a fixed quota” of medication that patients can get in Korea.

As an international student at UW-Madison, Kangwoo couldn’t get insurance through the Student Health Insurance Plan or SHIP.

Instead, he pays about \$600 a month for insurance through Quartz, formerly Unity, and is grateful that the cost is covered by Patient Service, a non-profit premium and co-payment assistance foundation that helps low-income people afford health insurance.

There is clotting factor in South Korea, but because of the cost and access issues, there are caps on how much factor a person there can receive, Lovell said.

“As you might imagine, the situation is far more dire in other countries where access to factor is severely limited,” she said.

Kangwoo, for his part, is grateful not only to his musical mentors, but to the medical staff that have been caring for him in Madison these past four years. He’d like to stay in the U.S., and is pursuing assistant professorship positions. Meanwhile, he’s teaching and performing and will go to Germany next month for a music festival and conference.

“Thankfully there are great people who are actually caring more of myself than myself,” Kangwoo said. “That’s really incredible in America.”